

**The Creating Hope Act: Market Incentives for the Development of Drugs
For Pediatric Cancer and Other Pediatric Rare Diseases**

Presentation to The Congressional Pediatric Cancer Caucus'
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NANCY F. GOODMAN
Founder and Executive Director, Kids v Cancer
NancyGoodman@KidsvCancer.org

Congressman McCaul, Congressman Van Hollen, Members of the Pediatric Cancer Caucus:
Thank you for inviting me to the Congressional Pediatric Cancer Caucus' Second Annual
Childhood Cancer Summit to talk about a crisis that is occurring in pediatric cancer -- the
absence of research and development for new drugs for our children.

And thank you and Congressmen and Congresswomen McCaul, Butterfield, Myrick and Van
Hollen for introducing the Creating Hope Act in the House of Representatives today.

JACOB'S STORY

I am here today because my son, Jacob, died two and a half years ago of a pediatric brain
cancer. Jacob was a beautiful 8 year-old boy with red hair and a brilliant, kind smile when he
was diagnosed. Overnight, he went from being a loud and precocious kid who loved to play any
sport with a ball, to a boy in a wheelchair who at times couldn't talk, who had serious cognitive
impairments and who couldn't eat or control his basic bodily functions. He spent 9 of the
remaining 23 months of his life inpatient in hospitals and much of the rest in and out of clinic,
but he carried his burden with a smile and good humor. He was so brave. A trip to the M&M
store in Times Square on the way home from a clinic visit was always more interesting to Jacob
than dwelling on how much he hurt, or when he'd have to go back for more treatment.

Today, if he hadn't been sick, Jacob would have been 13. He would have been bar mitzvahed
this year. I imagine he would have been tall and skinny, with a cracking voice. Perhaps he
would have complained about his writing homework. Perhaps he would still be in a rock band.
I know he would have spent hours a day talking to his younger brother, Ben. He would have
loved to have met his baby sister, Sarah.

If there had been effective drugs to treat Jacob, I would have had the chance to watch him
grow into a healthy young man instead of speculating like this. But two weeks after Jacob's
diagnosis, Jacob's doctors knew the drugs they intended to give him were not likely to work.
Yet they continued to use them because there were no alternatives. There have been no
material changes to treat Jacob's form of cancer in decades.

FACTS OF PEDIATRIC CANCER

Pediatric cancer is thought by some as an obscure, low impact cancer. Let me say this: pediatric cancer is not low impact. It has a profound effect. Cancer is the disease that kills the most children and young adults in the United States. Thousands of children die of cancer every year. They miss their whole lives when they die. And the lucky ones – the hundreds of thousands of survivors – they live with a raft of impairments and often life threatening conditions. Measured in years of life lost and the permanent loss of health for survivors, pediatric cancer's impact rivals that of breast cancer.

And the death of even one child has a great impact on families and communities. There's something particularly unjust about a kid dying of a disease that should be treatable. It just sticks with you.

I want to share with you a few of the things I learned about pediatric cancer through the experience of taking care of Jacob, talking to dozens of parents with sick children and consulting with literally scores of research institutions in search of better treatments.

What I learned is the following:

First, pediatric cancers are different from adult cancers in a number of ways. Kids don't get prostate cancer, breast cancer, or colon cancer. Childhood cancer's molecular biologies may be different from adult molecular biologies. Treatments are different. So it is not always the case that a drug developed for an adult cancer is always effective in children. Moreover, there are many instances in which a drug may benefit a child, but not an adult. These drugs never get developed.

Second, there have been no new drugs developed for pediatric cancer in many years. In fact, in the past 20 years, the FDA has initially approved only one drug for any childhood cancer.

Third, unless we do something, it is unlikely that new drugs will be developed for pediatric cancers in the future, either. Because the childhood cancer drug markets are so small, there are almost no drug companies undertaking research and development for new drugs.

Fourth, for a number of structural reasons I can go into at another time, at 4% of the National Cancer Institute budget, pediatric cancer research is underfunded by the government as well.

Now let me be clear: There has been progress. But some of the statistics can be misleading. The adult cancer community measures progress in terms of five-year survival rates, but improvements in five year survival rates don't really matter when your child dies in his 20's of a secondary cancer. And few of the long-term survivors achieve a "cure." The word cure implies a return to health. Our kids now get doses of highly toxic chemotherapies and radiation that

leave them scarred for life. So, perhaps more survive, but they survive much sicker – not because of the cancer, but because of the treatments themselves. Had Jacob survived, the treatments he received would have rendered him unable to live a normal and independent adult life.

Since Jacob's death, I founded Kids v Cancer to try and eliminate the obstacles that stand in the way of new drugs being developed to treat pediatric cancer and other pediatric rare diseases.

THE CREATING HOPE ACT AND INCENTIVES FOR CANCER DRUG DEVELOPMENT FOR CHILDREN

We live in the country with the world's leading researchers and scientists, and yet almost none of that talent is being directed toward drugs for our children. The problem is that the size of the markets for pediatric cancer drugs is too small for a private sector return on investment.

There have been a number of legislative efforts to promote new drug development, but frankly, they have not been effective for pediatric cancer drugs. Extending patent protection or market exclusivity for drugs expressly designed for pediatric cancer does not provide a sufficient incentive because the markets are too small for that patent protection to be valuable. If we really care about protecting our kids, we must create incentives that occur outside the markets of the pediatric cancer drugs. The Creating Hope Act does just that.

The Creating Hope Act builds on the neglected tropical disease priority review voucher by improving it and expanding it to include pediatric cancer and other pediatric rare diseases. In designing the Creating Hope Act, we've tried to take into account the fact that this is not the best time to propose an increased appropriation or a costly program. The legislation does not require any appropriation. I want to also note that the Creating Hope Act closes certain loopholes that could create a windfall for pharmaceutical companies.

Under the Creating Hope Act, if a pharmaceutical company gets all the way to FDA approval for a drug that addresses a pediatric cancer or other pediatric rare disease, a voucher would be awarded. The voucher would come with rights to a priority review for any other drug for which the voucher was exercised. This would result in the other drug being introduced to market more quickly once approved, creating significant value.

The Creating Hope Act requires real results for a real benefit.

I urge all Members of Congress to support this bill and shepherd it into law. If you've known a child who died – particularly of a condition that should be treatable – you know what a profound impact it has – including on the family and on the community.

We should not tolerate the deaths of thousands of children each year and condemnation hundreds of thousands more to lives of significant impairment. We should create a world in

which children with cancer do not have to suffer as Jacob did. We can do better. We must try. Creating Hope Act is one step in that direction.

Thank you.